ESGCT 2025 Clinical and AI / ML Preview

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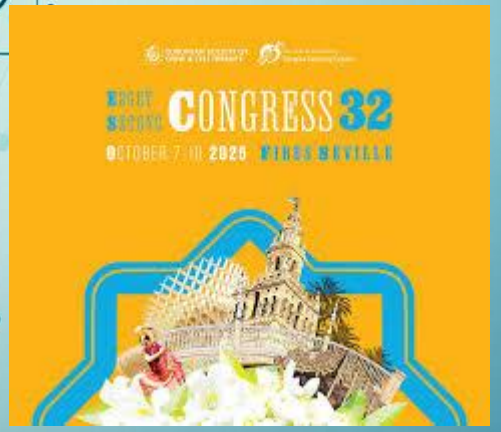

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ESGCT 2025 – General Overview

- **Next-Generation Gene & Cell Therapy Advances:** ESGCT 2025 is expected to emphasize new AAV capsid platforms, base-editing, and RNA technologies driving safer, more durable, and precisely targeted therapies across multiple organ systems
- **Clinical Momentum and Translation:** Presentations are anticipated to reveal updated data from long-term follow-up studies, early gene editing studies, and regenerative trials in various disorders
- **Computational and AI Integration:** Sessions will explore AI, machine learning, and modeling tools for optimizing vector design, biodistribution prediction, and manufacturing analytics, accelerating translation from discovery to clinic
- **Manufacturing Scalability and Quality:** Expect focus on automated, single-use bioreactor systems, digital process controls, and high-precision analytics, ensuring consistency and cost-efficiency for advanced therapy medicinal products
- **Global Access and Regulatory Innovation:** ESGCT 2025 will emphasize equitable access frameworks, evolving ATMP regulations, and coordinated EMA-FDA guidance supporting clinical adoption of curative gene and cell therapies

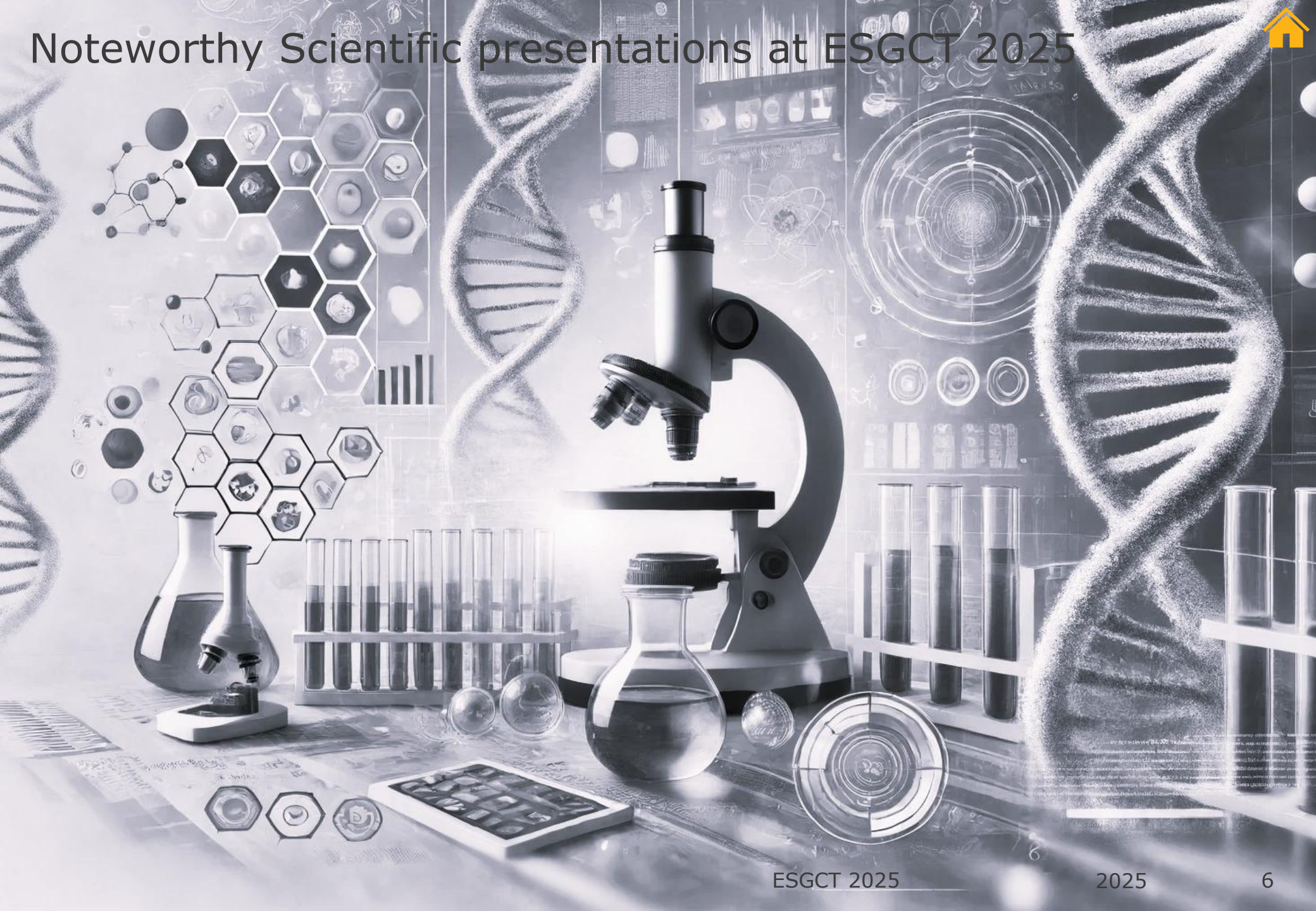
ESGCT 2025– Conference Themes (1/2)

- **Precision Gene Editing:** Focus on base, prime, and twin-prime editing tools, achieving refined genomic correction with enhanced specificity, efficiency, and durability across hematologic and neuromuscular disorders
- **Next-Wave Delivery Systems:** Spotlight on hybrid non-viral, LNP, and circular DNA systems designed for redosing potential, tissue selectivity, and scalable manufacturing compatibility
- **CNS and Sensory Restoration:** Advancements in gene therapies for Rett syndrome, SMA, and inherited retinal diseases will highlight breakthroughs in trans-barrier delivery and long-term functional outcomes
- **Hematopoietic Stem-Cell Platforms:** Deep dive into optimized conditioning, vector integration safety, and multi-enzyme correction strategies redefining HSC-based treatments for lysosomal and immune deficiencies

ESGCT 2025– Conference Themes (2/2)

- **AI-Driven Development:** Integration of artificial intelligence and deep learning for predictive modeling, vector optimization, and early-signal detection to enhance translational efficiency
- **Global Equity and Policy:** Discussion on reimbursement models, manufacturing localization, and public-private initiatives shaping equitable access to curative gene and cell therapies
- **Immunogenicity and Safety Profiling:** Enhanced pre-clinical frameworks to characterize immune responses, vector persistence, and long-term biodistribution for next-generation AAV and LV products
- **Convergent Therapeutic Modalities:** Exploration of synergistic platforms combining gene editing, RNA modulation, and cell-based regeneration to advance durable, multi-pathway disease correction

Noteworthy Scientific presentations at ESGCT 2025



Key Topics From Notable Presentations (1/4)

- **Liver-Directed & Metabolic Gene Therapies:** ESGCT 2025 is set to spotlight OTC-HOPE, ReGenHeart Phase 2, LY-M003, and GNT-012-CRIG studies, advancing durable correction in metabolic and hepatic disorders. Long-term follow-up data and AAV vector refinements for Wilson's, OTC deficiency, and Crigler-Najjar syndromes are anticipated to strengthen curative potential and improve safety-efficacy balance in systemic delivery
- **Ocular & Sensory System Gene Therapies:** Ophthalmic programs such as laru-zova (DAWN Trial), PRODYGY (RdCVF), and CTx001 for dry AMD are expected to dominate this track. Novel AAVs targeting DFNB1A deafness and Stargardt disease will enhance retinal rescue strategies, while gene therapies for inherited blindness expand translational precision and optimize subretinal versus intravitreal administration paradigms
- **Central Nervous System & Neurodegenerative Disorders:** Key Presentations are expected to highlight NGN-401 for Rett syndrome, MECP2 editing, and acid sphingomyelinase deficiency programs. Gene therapies for Alzheimer's, Wolfram, and Angelman syndromes will demonstrate cross-species vector validation and optimized CNS biodistribution, establishing future standards for durable neurological correction, one-time dosing, and ethical design in brain-targeted interventions

Key Topics From Notable Presentations (2/4)

- **Hematopoietic Stem Cell & Inherited Hematologic Disorders:** Clinical updates on OTL-203, ARTEGENE Phase I/II, and RAG1-SCID are anticipated to underscore durable engraftment and biochemical correction. New preclinical data in MPS IVA and α-Mannosidosis models will demonstrate improved vector copy control and reduced conditioning toxicity, advancing ex vivo HSPC therapy as a safe, scalable curative platform
- **Neuromuscular & Musculoskeletal Disorders:** Programs such as GEN6050X (DMD Base Editor), Optidys dual-AAV, and LMNA-related CMD are projected to lead this area. The RIDGETM-1 (TN-401) study for PKP2-ARVC and novel α-Klotho-based strategies will enhance skeletal and cardiac muscle function, while long-term durability in GRMD dogs informs translatable dosing strategies
- **Cardiovascular, Renal, and Metabolic Disorders:** Gene therapies, including CBN-1100, NVC-001, and FGF21, are anticipated to reshape cardiovascular disease treatment. AAV9 platforms for cardiomyopathy and renal pathologies accelerate precision dosing and enable first-in-class curative interventions for heart-metabolic spectrum disorders

Key Topics From Notable Presentations (3/4)

- **Oncology, CAR-T, and Oncolytic Virus Therapies:** Trials such as AloCELYVIR Phase Ib, TILT-123, and HER2 sdAb-CAR-T are expected to define new standards in solid-tumor immunotherapy. AI-enhanced CAR engineering, dual-payload oncolytic viruses, and combination checkpoint regimens will likely expand tumor selectivity, persistence, and real-world translational readiness for next-generation onco-viral precision therapy
- **Emerging Technologies & Non-Viral Delivery:** Key innovations will include FiCAT, Megabulb DNA, and UCOE constructs, bridging CRISPR-Cas precision with scalable manufacturing. Novel LNPs, extracellular vesicles, and programmable RNA systems are expected to outperform viral modalities in safety and payload versatility, paving the way for modular, repeat-dose gene and mRNA therapeutics
- **Manufacturing, Platforms & Analytical Innovation:** Sessions featuring iCellis500, Refeyn SamuxMP, and Recombumin® will redefine process scalability and quality. Topics will address high-fidelity capsid analytics, digital PCR trace detection, and GMP upstream automation, supporting regulatory compliance and cost-efficient global rollout of advanced therapy manufacturing ecosystems

Key Topics From Notable Presentations (4/4)

- **Regulatory, Access & Clinical Development:** Policy discussions on Join4ATMP, Holoclar® Phase IV, and EMA–FDA harmonization will shape ATMP access pathways. Sessions will examine equitable global trial inclusion, long-term safety registries, and adaptive licensing frameworks critical for accelerating patient reach and maintaining ethical oversight of next-generation gene-editing therapies

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (1/8)

- **Viralgen:**
  - Focus Areas: Advanced AAV Capsid Design & CNS-Targeted Gene Editing
  - Viralgen is going to present its next-generation AAV engineering platforms showcasing RNA-based StitchR/CirculR technologies, BBB-crossing vectors, and capsid innovations expected to transform CNS delivery, neuromuscular targeting, and epigenetic silencing applications
- **Lonza:**
  - Focus Areas: In Vivo & Non-Viral CAR-T Engineering
  - Talks will feature lentiviral and DNA-based vectors for in vivo CAR-T generation, dual-target STAb-T cells, antigen-specific Tregs for autoimmune disease, and cell-free DNA profiling to monitor CAR-T efficacy and safety

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (2/8)

- **PlasmidFactory:**
  - Focus Areas: Non-Viral Vector Platforms & Transposon-Based CAR-T Manufacturing
  - PlasmidFactory is going to highlight innovations in Sleeping Beauty and Minicircle platforms, showcasing non-viral CAR-T and TCR-T manufacturing advances designed to improve safety, scalability, and cost-efficiency for solid-tumor and autologous applications
- **ERC-EIC:**
  - Focus Areas: Translational Innovation & European Funding Strategies
  - Sessions will provide overviews of ERC and EIC mechanisms accelerating academic-industry translation in cell and gene therapy, highlighting frameworks that support frontier research moving into early-stage clinical programs

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (3/8)

- **Sartorius:**
  - Focus Areas: CGT Process Optimization & Cost Efficiency
  - "Walking the Tightrope" will discuss balancing process robustness with cost reduction to expand cell- and gene-therapy accessibility through integrated manufacturing analytics and adaptive bioprocess control
- **Revvity:**
  - Focus Areas: Next-Generation Gene Editing for Rare Diseases
  - Updates will include CRISPR/Cas9 programs for ATTR and HAE, gene therapies for DMD and Tay-Sachs/Sandhoff, and platform advances bringing editing therapies closer to market readiness and regulatory validation

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (4/8)

- **Accuredit Therapeutics:**
  - Focus Areas: Single-Dose Gene Editing for Lipid Disorders
  - Data on ART002 will demonstrate durable LDL-C reduction in heterozygous familial hypercholesterolemia, underscoring safety, precision, and the potential for functional cure after one administration
- **Roche:**
  - Focus Areas: Immune Modulation to Enable AAV Redosing
  - Roche will present its approach to transient immune suppression via CD28/B7 pathway modulation, illustrating strategies to extend AAV therapy eligibility and improve redosing flexibility in future systemic applications

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (5/8)

- **Vivet Therapeutics:**
  - Focus Areas: Overcoming AAV Immunity
  - Vivet Therapeutics is expected to showcase VTX-PID (imlifidase)-based solutions for AAV neutralizing-antibody removal, demonstrating translational advances supporting broader patient inclusion and optimized dosing designs for AAV3B-driven programs
- **AstraZeneca:**
  - Focus Areas: Precision Genome Editing
  - AstraZeneca is preparing to highlight its SpOT-On precision genome-editing platform, expected to demonstrate enhanced targeting accuracy and improved efficiency for future in-vivo therapeutic applications across metabolic and monogenic diseases

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (6/8)

- **Merck:**
  - Focus Areas: AAV Manufacturing Scale-Up
  - To 1000 L and Beyond" will outline innovations in upstream AAV production, emphasizing yield optimization, cost reduction, and process robustness for next-generation large-scale bioreactors
- **Galapagos BV:**
  - Focus Areas: Decentralized Cell Therapy Manufacturing
  - The Galapagos platform will highlight distributed manufacturing networks enabling flexible, point-of-care autologous cell-therapy production to accelerate patient access

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (7/8)

- **Cytiva:**
  - Focus Areas: Large-Scale T-Cell Expansion Systems
  - Data on Xuri™ W25 systems will demonstrate lipid-nanoparticle-assisted transfection for T-cell therapies, improving efficiency and scalability in CGT workflows
- **CSL Behring:**
  - Focus Areas: Hemophilia & Liver Safety Outcomes
  - CSL Behring is preparing to discuss updates on etranacogene dezaparvovec and hemophilia B AAV therapies, focusing on next-generation liver-safety monitoring and the pathway toward regulatory submission and long-term therapeutic durability

Focus of Key Industry-Sponsored Sessions at ESGCT 2025 (8/8)

- **AskBio:**
  - Focus Areas: Multi-Indication AAV Clinical Pipeline
  - Highlights include RGX-202 for DMD, LUCE Phase I/II for Usher 1B retinitis pigmentosa, RESET-PV (resecabtagene autoleucel) CAR-T for pemphigus vulgaris, and GNT0004 (GNT-016-MDYF) long-term follow-up in DMD
- **CRISPR-X / CRISPR Therapeutics:**
  - Focus Areas: In-Vivo Gene Correction with LNP Delivery
  - Sessions will spotlight single-dose SyNTase editor technology achieving precise AATD correction, emphasizing RNA-guided editing efficiency and clinical translatability

Notable Presentations And Late-breaking Sessions At ESGCT 2025

Notable Presentations Information At ESGCT 2025 Cardiovascular, Renal, and Metabolic Disorders (1/2)

Date	Author	Title
08 Oct 2025	S Milagros	ADPKD gene therapy: Identification and characterization of recombinant adenoassociated virus-based vectors with renal tropism
08 Oct 2025	Hiroyuki Nakai	Defining therapeutic thresholds in AAV gene therapy for Alport syndrome via AI-based quantification of COL4A5 deposition in the glomerular basement membrane
08 Oct 2025	Sebastian Aguirre Kozlouski	Advancing cardiovascular gene therapy with CBN-1100: A next-generation vector platform for enhanced precision and safety
08 Oct 2025	Natasha Paterson	RIDGETM-1: a phase 1b interventional study to evaluate safety and efficacy of TN-401, an adeno-associated virus serotype 9 (AAV9) investigational gene therapy, in adults with PKP2-associated arrhythmogenic right ventricular cardiomyopathy (ARVC)
08 Oct 2025	Benedetta Ruzzenente	AAV9-based gene therapy prevents mitochondrial dysfunction and cardiomyopathy in a mouse model of LRPPRC deficiency
08 Oct 2025	Seppo Yla Herttuala	Adenoviral vascular endothelial growth factor-DΔNΔC gene therapy improves clinical symptoms, exercise tolerance and health-related quality of life in refractory angina pectoris patients. ReGenHeart Phase 2 trial
09 Oct 2025	Yann Chong Tan	NVC-001 - An AAV Gene Therapy Functional Cure for LMNA Dilated Cardiomyopathy
09 Oct 2025	Anna Katharina Schrattel	Cross-Species Selected Synthetic AAVs For Targeted Cardiac Gene Therapy

Notable Presentations Information At ESGCT 2025 Cardiovascular, Renal, and Metabolic Disorders (2/2)

Date	Author	Title
09 Oct 2025	Ester López-Aguilar	Inhibition of PCSK9 with Polypurine Reverse Hoogsteen hairpins: a novel gene therapy approach
09 Oct 2025	C Binny	FGF21 gene therapy: A single administration treatment strategy to treat severe obesity and associated comorbidities
10 Oct 2025	Jonathan Schwartz	Transformative Treatment of Devastating Cardiomyopathies: Safety and Efficacy in AAV Clinical Development of RP-A501 (Danon Disease) and RP-A601 (PKP2-ACM)

Notable Presentations Information At ESGCT 2025
Central Nervous System & Neurodegenerative Disorders (1/2)

Date	Author	Title
08 Oct 2025	Alice Arnould	Characterizing the tissue specific epigenetic regulation of the AAV9-SMN1 gene therapy for Spinal Muscular Atrophy (SMA).
08 Oct 2025	Cristina Sánchez-Puelles	E2F4-based gene therapy restores long-term potentiation and memory formation and blocks neuronal death in an Alzheimer mouse model
08 Oct 2025	Heather Born	Nonclinical Efficacy of MVX-220, an AAVhu68-based Investigational Gene Therapy, in a Mouse Model of Angelman Syndrome (AS)
08 Oct 2025	IM Revers	Brain-directed gene therapy rescues a mouse model of neurodegenerative vanishing white matter disease
08 Oct 2025	Ángela Sánchez Osuna	Gene therapy strategy for the treatment of Wolfram Syndrome
08 Oct 2025	Daniel Virga	Development of a Durable Gene Therapy for Targeting CNS and Visceral Pathologies in Acid Sphingomyelinase Deficiency
08 Oct 2025	Francesca Morosi	Base-editing mediated MECP2 gene correction, a new gene therapy strategy for Rett Syndrome patients.
08 Oct 2025	Françoise Piguet	Combine intravenous gene therapy overexpressing CYP46A1 and BBB opening can rescue Parkinson disease in a NHP induced PD model

Notable Presentations Information At ESGCT 2025
Central Nervous System & Neurodegenerative Disorders (2/2)

Date	Author	Title
09 Oct 2025	Ricardo Reis	AAV9-ERCC6 delivery as a potential gene therapy for Cockayne Syndrome
09 Oct 2025	Laura Rodriguez-Estevez	Hereditary Spastic Paraplegia Type 52 (SPG52): Gene Therapy Strategy
09 Oct 2025	Lucía Medialdea Ortiz	Use of brain organoids to study the safety and efficacy of nanosytems targeting central nervous system
09 Oct 2025	A Behr	Development of a gene therapy strategy for GNB1 Encephalopathy: Construction of a silence-repair system
09 Oct 2025	Nicole Jastrzebowska	Haematopoietic stem cell gene therapy for people with Alzheimer's Disease.
09 Oct 2025	Suzanne R Burstein	Route matters: intracerebroventricular (ICV) delivery of NGN-401 drives superior transgene expression to key areas of the brain when compared to intrathecal (IT-L) delivery at clinically relevant dose — implications for a one-time gene therapy for Rett syndrome
09 Oct 2025	Laetitia Heng	Development of a gene therapy based on SMaRT technology for Huntington's disease

Notable Presentations Information At ESGCT 2025
Emerging Technologies & Non-Viral Delivery (1/2)

Date	Author	Title
08 Oct 2025	Zoe Walker	VMiX™: A novel AAV-based RNA interference platform demonstrating consistent gene silencing efficacy in mouse studies
08 Oct 2025	YW Son	Localized Gene Therapy : AAV variants designed for smooth muscle cell transduction
08 Oct 2025	Prassanna Rao	Exploring the Role of Inverted Terminal Repeats in AAV Vector Performance for CNS Gene Therapy
08 Oct 2025	Trevor Smith	Employment of the CELLECTRA® in vivo gene delivery platform in a first in human (FIH) DNA encoded monoclonal antibody (DMAb) clinical trial
08 Oct 2025	K Wolter	Biodistribution of lipid nanoparticles for in vivo delivery of mRNA for gene therapy
08 Oct 2025	L de Lemos	Efficient non-viral delivery of mRNA to the mouse retina: a strategy for gene therapy in Choroideremia
08 Oct 2025	Francesco Ragazzini	Synthetic Protein-Level Feedback Circuits Enable Safe Gene Dosage Control for Gene Therapy
08 Oct 2025	Dario Gajewski	Non-viral gene therapy fully rescues osteoclast function in a stem cell model of osteopetrosis

Notable Presentations Information At ESGCT 2025
Emerging Technologies & Non-Viral Delivery (2/2)

Date	Author	Title
09 Oct 2025	Elena Stoyanova	Enabling gene therapy with Megabulb DNA -- a novel circular single-stranded CRISPR editing template
09 Oct 2025	Omer Anakok	A smaller, stable and higher expression UCOE model for gene therapy and recombinant production applications
09 Oct 2025	Matthew Dale	Programmable RNA Control Systems for Tuneable Regulation of Gene Therapy Activity

Notable Presentations Information At ESGCT 2025
Hematopoietic Stem Cell & Inherited Hematologic Disorders (1/2)

Date	Author	Title
07 Oct 2025	Maria Ester Bernardo	Extensive metabolic detoxification and favorable clinical outcomes sustained through 5 years post-treatment in Hematopoietic Stem Cell Gene Therapy (OTL-203) for Mucopolysaccharidosis Type I-Hurler
08 Oct 2025	Susana Navarro Ordonez	Toward Clinical Translation of Lentiviral Gene Therapy for Diamond-Blackfan Anemia: Preclinical Studies Targeting RPS19
08 Oct 2025	Beatriz Olalla Sastre	Improving Prime Editing efficacy for Hematopoietic Stem and Progenitor Cells targeting
08 Oct 2025	Saranya Elavazhagan	Preclinical Safety and Effective Dose Modelling of an Autologous Haematopoietic Stem Cell Gene Therapy for the Treatment of NOD2-Deficient Severe Crohn's Disease
08 Oct 2025	G Pedrazzani	Overcoming toxic conditioning in hematopoietic stem cell gene therapy leveraging mobilization-resistant CXCR4 variants
08 Oct 2025	Luca Seffin	Investigating the biological properties of circulating Hematopoietic Stem/Progenitor Cells in pediatric subjects as novel source for Gene Therapy.
08 Oct 2025	Ying Hong	Design and validation of a GMP stem cell manufacturing protocol for Deficiency of adenosine deaminase 2 (DADA2) gene therapy
08 Oct 2025	Bruno Salomone Gonzalez de Castejon	Haematopoietic stem cell gene therapy for ataxia-telangiectasia

Notable Presentations Information At ESGCT 2025
Hematopoietic Stem Cell & Inherited Hematologic Disorders (2/2)

Date	Author	Title
08 Oct 2025	Angela Gritti	Myeloid-mediated enzymatic correction of ARSA-deficient neural cells in hematopoietic stem cell gene therapy for metachromatic leukodystrophy
08 Oct 2025	Brian Bigger	Long-term haematopoietic stem cell gene therapy in Mucopolysaccharidosis IIIB mice corrects disease with no evidence of insertional mutagenesis despite high vector copy numbers
08 Oct 2025	Rachele D'Amore	Pre-clinical development of an ex-vivo Hematopoietic Stem/progenitor Cells-gene therapy for a-Mannosidosis
09 Oct 2025	Christina Beta	Hematopoietic Stem Cell -- based gene therapy for Friedreich's Ataxia: From ex vivo optimization to in vivo Assessment
09 Oct 2025	Gaia Alberti	Preclinical development of Hematopoietic Stem/Progenitor Cells (HSPC)-gene therapy (GT) for GLB1 related disorders in a newly characterized Glb1-/- mouse model
09 Oct 2025	Selene Ceriotti	Preclinical development of lentiviral-based hematopoietic stem/progenitor cells (HSPC)-gene therapy (GT) for Mucopolysaccharidosis type IVA (MPSIVA) as part of an innovative GT platform approach for LSDs.
09 Oct 2025	Rachel Searle	Anti-SGSH antibodies following Hematopoietic Stem Cell (HSC) Gene Therapy in Patients with MPSIIIA Neither Impact Engraftment of Genetically Modified HSC nor Interfere with Multi-compartment Substrate Reduction
10 Oct 2025	Rob Wynn	Hematopoietic stem cell and stem cell gene therapy in metabolic diseases

Notable Presentations Information At ESGCT 2025
Liver-Directed & Metabolic Gene Therapies (1/3)

Date	Author	Title
07 Oct 2025	Gloria Gonzalez-Aseguinolaza	Gene addition vs gene editing strategies in liver-directed gene therapy
07 Oct 2025	Pasquale Piccolo	Overcoming the challenges of gene therapy in inborn error of metabolism with liver damage
07 Oct 2025	George Diaz	OTC-HOPE: The first in vivo, liver directed, AAV-mediated, gene insertion clinical trial in infants
07 Oct 2025	Elena Barbon	Correction of glycogen storage disease type Ia by in vivo liver-directed lentiviral gene therapy in a mouse model
08 Oct 2025	Natalia Artigas	Performance and improvements of FiCAT as a gene therapy for Haemophilia A in vivo
08 Oct 2025	Andrei Claudiu Cozmescu	Advancement of lentiviral gene therapy for progressive familial intrahepatic cholestasis type 3
08 Oct 2025	Andrew Lin	Revolutionizing Wilson's Disease Treatment: Clinical Safety and Efficacy of LY-M003, A Copper-Responsive AAV Gene Therapy Vector
08 Oct 2025	Jiake Zhong	Gene therapy vs. cochlear implantation in restoring hearing function and speech perception for congenital deafness individuals

Notable Presentations Information At ESGCT 2025
Liver-Directed & Metabolic Gene Therapies (2/3)

Date	Author	Title
09 Oct 2025	Gloria González-Aseguinolaza	Evaluation of Liver-Directed Gene Therapy in mice with Cerebrotendinous Xanthomatosis Pre-treated with Chenodeoxycholic Acid
09 Oct 2025	C Smerdou	Liver-directed gene therapy results in long-term amelioration of progressive familial intrahepatic cholestasis type 2 in juvenile mice
09 Oct 2025	Giovanna Giacca	From mice to men: translating in vivo gene therapy for liver metastases in a novel hemato-chimeric mouse model hosting human liver-resident macrophages
09 Oct 2025	Lindsey George	Next Generation Hemophilia A Gene Therapy
09 Oct 2025	Graham Foster	Liver related problems with gene therapy – what can we do to mitigate them?
10 Oct 2025	Paul Gissen	AAV-mediated gene therapy for OTC deficiency
10 Oct 2025	Jeremy Do Cao	Overcoming AAV8 Immunity: First Seropositive Crigler-Najjar Patient Treated with GNT0003 Following Imlifidase Pretreatment (GNT-018-IDES clinical trial)
10 Oct 2025	Lorenzo D'Antiga	Durability of AAV-based gene therapy in patients with Crigler Najjar syndrome at 4 years of follow up after dosing: the GNT-012-CRIG Study

Notable Presentations Information At ESGCT 2025
Liver-Directed & Metabolic Gene Therapies (3/3)

Date	Author	Title
10 Oct 2025	Giuliana Ferrari	Gene therapy for beta-thalassemia: towards the new academic trial
10 Oct 2025	Maria Battipaglia	Modulation of TRPML1/TFEB pathway for the treatment of Wilson Disease
10 Oct 2025	Rachel Searle	Anti-SGSH antibodies following Hematopoietic Stem Cell (HSC) Gene Therapy in Patients with MPSIIIA Neither Impact Engraftment of Genetically Modified HSC nor Interfere with Multi-compartment Substrate Reduction
10 Oct 2025	C Smerdou	Liver-directed gene therapy results in long-term amelioration of progressive familial intrahepatic cholestasis type 2 in juvenile mice

Notable Presentations Information At ESGCT 2025 Manufacturing, Platforms & Analytical Innovation (1/2)

Date	Author	Title
08 Oct 2025	Chiara Bresesti	Comet LV: a lentiviral vector-based mRNA co-packaging technology for enhanced ex vivo and in vivo gene therapy
08 Oct 2025	IRS Ferreira	Raising the Bar: Improved Reference Standards for rAAVs in Gene Therapy
08 Oct 2025	Mathias Kahl	iCellis500: A Versatile Manufacturing Platform for Cell & Gene Therapy (CGT) and Oncolytic Virus Production - Lessons Learned and Best Practices from Successful Programs
08 Oct 2025	Claire Gueguen	Advancing Gene Therapy: Stabilization of Viral Formulations with Recombumin® Human Albumin
08 Oct 2025	Clothilde Decarnin	Optimizing Detergent Use for AAV Recovery and Viral Inactivation in Gene Therapy Manufacturing
08 Oct 2025	Clotilde Ciesla	Accelerating small scale development in gene therapy: Fast and robust full capsids quantification using Mass Photometry Technology from Refeyn (SamuxMP).
08 Oct 2025	Silvia Gomez	A Platform Approach for Streamlining and Accelerating Gene Therapy Commercialization
08 Oct 2025	erik Hansen	AnelloBricks®: Development of a Scalable, Low-Cost, In-Vitro Assembled Anellovirus-Derived Platform for Gene Therapy Applications

Notable Presentations Information At ESGCT 2025 Manufacturing, Platforms & Analytical Innovation (2/2)

Date	Author	Title
08 Oct 2025	Atsushi Iesato	Implementing Virus Filtration to Ensure Pathogen Safety of Cell and Gene Therapy Products
08 Oct 2025	Sana Ahmed-Seghir	Traces matter : high-sensitivity dPCR assays for residual host DNA monitoring in Cell and Gene therapy & Bioproduction
09 Oct 2025	I Weber	Enhancing rAAV Vector Production: A Stable Cell Line Approach to Overcome Manufacturing Barriers in Gene Therapy
09 Oct 2025	Carles Celma	A Unified Analytical Framework for the Characterization of AAV Vectors in Gene Therapy
09 Oct 2025	Kathrin Wenger	Evaluation and optimization of different digestion strategies for in-depth proteomic characterization of residual host cell proteins in rAAV-based gene therapy products
09 Oct 2025	Claudia Labrador Rached	High-Sensitivity Mycoplasma Detection Using Crystal Flex Probe Technology and Digital PCR: A New Standard for Cell and Gene Therapy Workflows
09 Oct 2025	Femke Hoeksema	An insect cell based scalable platform for AAV production for gene therapy

Notable Presentations Information At ESGCT 2025
Neuromuscular & Musculoskeletal Disorders (1/2)

Date	Author	Title
07 Oct 2025	Sergi Verdes	Targeting Skeletal Muscle to protect Motor Neurons: α-Klotho Gene Therapy acts through NMJ Signaling in ALS
08 Oct 2025	Ina Luksch	AAV-mediated gene therapy in a porcine model of Duchenne Muscular Dystrophy
08 Oct 2025	Caroline Le Guiner	11-year of efficacy of a single administration of AAV8 microdystrophin gene therapy in a GRMD dog
08 Oct 2025	Andrea Bähr	Transcriptional map of gene therapy in a PLN R14del pig model
08 Oct 2025	Theresa Schmid	Precision-Targeted Expression: A Novel Synthetic Promoter for Skeletal Muscle Gene Therapy
08 Oct 2025	Matthew Borok	Derivation of satellite cell-specific AAV gene therapy
08 Oct 2025	Ignacio Perez de Castro	Pioneering gene therapy approaches for LMNA-related congenital muscular dystrophy
08 Oct 2025	Chunyan He	Positive motor and cardiac function improvements of DMD Base editor, GEN6050X, in early phase I study

Notable Presentations Information At ESGCT 2025
Neuromuscular & Musculoskeletal Disorders (2/2)

Date	Author	Title
08 Oct 2025	Badih salman	Mtm1-deficient rats as a new preclinical model for myotubular myopathy gene therapy
09 Oct 2025	Ai Vu Hong	A second-generation myotropic capsid targeting integrin alpha V beta 6 with enhanced transduction efficacy in skeletal and cardiac muscles
09 Oct 2025	Sumitava Dastidar	Assessing spatio-temporal dynamics of AAV transduction and therapeutic efficacy for neuromuscular disorders in a human multi-organoid system
09 Oct 2025	Sonia Albini	Optidys: a dual-AAV gene therapy strategy for Duchenne muscular dystrophy
09 Oct 2025	Déborah Gómez-Domínguez	Challenges and successes of LMNA overexpression as a potential gene therapy for L-CMD: a tale of two phenotypes
09 Oct 2025	Sebastian Logemann	Establishing a gene therapy approach for BAG3P209L myofibrillar myopathy

Notable Presentations Information At ESGCT 2025
Ocular & Sensory System Gene Therapies (1/2)

Date	Author	Title
07 Oct 2025	Virginia Haurigot	Gene therapy for the sensory systems (vision and audition)
07 Oct 2025	Hanen Khabou	Development of SPVN20, a gene therapy for cone reactivation in inherited retinal degeneration
08 Oct 2025	Mara Uhl	Comparing Dual AAV vs. Overload AAV approaches for Otoferlin gene therapy.
08 Oct 2025	Victor Guedes de Araujo	Promoter-dependent durability of hMAX gene therapy for long-term retinal ganglion cell protection in a glaucoma model
08 Oct 2025	S Dadak	In vitro assessment of functional gap junctions for novel AAV-based GJB2 gene therapy developed to treat DFNB1A deafness
08 Oct 2025	D Chung	Subretinal gene therapy laru-zova for X-linked retinitis pigmentosa (XLRP): Phase 2 DAWN Trial, preliminary month 9+ results
08 Oct 2025	Laure Blouin	PRODYGY: A first-in-human trial of rod-derived cone viability factor (RdCVF) gene therapy in subjects with rod-cone dystrophy
08 Oct 2025	A Didangelos	CTx001 an AAV2 Mini-CR1 Gene Therapy for dry AMD: IND-Enabling Studies Show Potent Complement Target Engagement and MAC Inhibition on Activated RPE Cells

Notable Presentations Information At ESGCT 2025
Ocular & Sensory System Gene Therapies (2/2)

Date	Author	Title
08 Oct 2025	Sheldon DECOMBE	Optimized intracochlear delivery strategies for gene therapy in hearing loss: A comparative preclinical study
08 Oct 2025	Paul Yang	Safety and efficacy of a phase 1/2 clinical trial for AAV5-mediated retinal gene augmentation of GUCY2D in Leber congenital amaurosis 1
09 Oct 2025	Stylianos Michalakis	Development and translation of a dual AAV gene therapy for ABCA4-associated Stargardt disease
09 Oct 2025	SA Fjeldstad	Retinal organoids as a preclinical model for AAV-based gene therapy for JNCL disease
09 Oct 2025	S Dadak	Safety and efficacy of GJB2-GT, an adeno-associated vector-based gene therapy treatment candidate for the autosomal recessive non-syndromic deafness 1A (DFNB1A)
09 Oct 2025	ALICE LE MEUR	Characterization of retinal degeneration in patients with rod-cone dystrophy to inform on potential efficacy endpoints of gene therapy

Notable Presentations Information At ESGCT 2025 Oncology, CAR-T, and Oncolytic Virus Therapies (1/2)

Date	Author	Title
08 Oct 2025	Alvaro Morales-Molina	Phase Ib clinical trial assessing safety, tolerability, and preliminary efficacy of AloCELYVIR (allogeneic mesenchymal cells + oncolytic adenovirus) in pediatric patients with recurrent/progressive medulloblastoma
08 Oct 2025	Patricia Garcia-Rodriguez	Synergistic immunotherapy for osteosarcoma: enhancing NKG2D-CAR T cell efficacy with armed oncolytic adenoviruses
08 Oct 2025	C Barbero-Jimenez	Novel HER2-Specific sdAb-CAR-T Cells Exhibit Enhanced Antitumoral Efficacy in HER2-Positive Cancers
08 Oct 2025	Munzur Lacin	Developing extracellular vesicles as next-generation gene therapy vectors for the treatment of glioblastoma
08 Oct 2025	BÜŞRA CESUR-ERGÜN	RGD-Modified Lipid-Polymer Hybrid Gene Delivery System for Enhanced Anti-VEGF Efficacy in Triple Negative Breast Cancer
08 Oct 2025	Lisa-Marie Dawson	In vitro and in vivo efficacy of novel "armed" oncolytic viruses derived from adenovirus species B for breast cancer therapy
08 Oct 2025	Johannes Frasez Soerensen	Delivering a CRISPR-based gene therapy targeting acute myeloid leukemia with RUNX1::RUNX1T1 to leukemic cells using lipid nanoparticles -- A proof-of-principle study
09 Oct 2025	Che Serguera	An amino acid-sensitive system for enhanced CAR-T cell efficacy and safety in solid tumours

Notable Presentations Information At ESGCT 2025 Oncology, CAR-T, and Oncolytic Virus Therapies (2/2)

Date	Author	Title
09 Oct 2025	Ana Hinckley-Boned	Enhancing CAR-T therapy for HER2+ tumors: physiological regulation for improved efficacy and safety
09 Oct 2025	V Salido-Subiñas	Cathepsin D and B impact in the anti-tumor efficacy of CART-BCMA cells against Multiple Myeloma
09 Oct 2025	Santeri A Pakola	Oncolytic adenovirus encoding TNF and IL2 (TILT-123): from preclinical development to clinical trials with tumor infiltrating lymphocyte therapy and checkpoint inhibitors
09 Oct 2025	Steven Pollard	Synthetic super-enhancers enable selective expression of anti-cancer payloads for viral gene therapy
09 Oct 2025	Saint T. Cervera Mayor	Ewing-specific GGAA promoter-based suicide gene therapy for Ewing sarcoma treatment

Notable Presentations Information At ESGCT 2025 Regulatory, Access & Clinical Development (1/2)

Date	Author	Title
07 Oct 2025	Annette Kunkele	Results of the Join4ATMP survey on ATMP development and access
08 Oct 2025	Charlotte Van Isterdael	Navigating regulatory challenges in the initiation of clinical trials for advanced therapy medicinal products (ATMPs): a scoping review
08 Oct 2025	Kelly Rendon	Global regulatory frameworks for clinical trials involving ATMPs: a literature review
08 Oct 2025	Michiel Vanhaeren	Advanced Therapy Medicinal Products are coming of age: a pipeline analysis of the clinical trial landscape
08 Oct 2025	FR Lopes	CAPACITY building on gene therapy clinical trials in East Africa
09 Oct 2025	Aimee Donald	Is the Early-Stage Gene Therapy Investigator as Well-Informed as they Could Be?
09 Oct 2025	Alix Wasilenko	Dynamics of Evidence Standards in Rare Disease Regulation: A Comparative Analysis of FDA and EMA Approaches to Gene Therapy Medicinal Products
09 Oct 2025	Alessia Merra	HOLOCORE lessons: biological and clinical insights from a pan-European phase IV clinical trial of Holoclar® for treating Limbal Stem Cell Deficiency

Notable Presentations Information At ESGCT 2025 Regulatory, Access & Clinical Development (2/2)

Date	Author	Title
09 Oct 2025	Stefano Benvenuti	Fondazione Telethon current strategies and future perspectives on patient access to gene therapy for ultra-rare disease
10 Oct 2025	James Wilson	Lessons learned from 35 years of in vivo gene therapy research
10 Oct 2025	Matthias Renner	First-in-human clinical trials: Regulatory considerations on manufacturing and control

Key Industry Sponsored Sessions Information

ESGCT 2025 Key Industry Sponsored Sessions Information (1/7)

Date	Sponsor	Title
07 Oct 2025	Viralgen	Toward a one time epigenetic silencing treatment for prion disease with BBB crossing AAVs
		StitchR and CircuIR: From RNA Stitching Technologies to Therapeutics
		Engineered AAV5 capsid SHP-DB1 efficiently targets the NHP brain after intravenous injection and transduces >95% of neurons in the Parkinson's disease-critical substantia nigra
		Covalent Antibody Conjugation to AAV9 for Targeted Gene Therapy of High-Risk Neuroblastoma
		Generation of a novel capsid engineering platform enhances CNS and muscle tropism of AAV vectors while reducing tropism to the liver
		An AAV6 variant engineers human resting T cells in vivo
		AAV-WM04, a Novel High-Efficiency Vector, Achieves Low-Dose Therapeutic Efficacy in Preclinical and Clinical Studies of OTOF-Related (DFNB9) Hearing Loss

ESGCT 2025 Key Industry Sponsored Sessions Information (2/7)

Date	Sponsor	Title
07 Oct 2025	Viralgen	Validation of Systemic AAV-PHP.eB-Mediated Delivery of Cholesterol Hydroxylase CYP46A1 as a Non-Invasive Gene Therapy Approach for Spinocerebellar Ataxia Type 3
07 Oct 2025	Lonza	Combined silencing of MED12 and activation of IL2 by epigenetic editing enhances CAR T antitumor potency.
		In Vivo CAR-T Cell Therapy using Engineered Lentiviral Vectors: From Concept to Clinic
		Epigenetic adjuvants for in vivo dendritic cell reprogramming
		Novel non-viral DNA-based gene therapy vector for in vivo CAR T engineering: insights into CAR-T persistence and biodistribution
		Development of antigen-specific Treg-based therapeutic products for Type 1 Diabetes
		Development of dual-targeted STAb-T cells for cancer immunotherapy

ESGCT 2025 Key Industry Sponsored Sessions Information (3/7)

Date	Sponsor	Title
07 Oct 2025	Lonza	Engineering Exhaustion-Resistant T Cells with IL-2 Variant (IL-2v) and CD40 Agonists to Enhance Antitumor Immunity
		Unveiling CAR-T Therapy Efficacy and Toxicity Through Cell-Free DNA Profiling
08 Oct 2025	PlasmidFactory	Welcome and brief introduction on PlasmidFactory's Minicircle
		Brief introduction on Minicircle-based CAR-T cells in clinical applications
		Enhanced efficiency of Sleeping Beauty transposon engineering of therapeutic cells by Minicircle vectors
		Non-viral manufacturing of tumor-specific TCR-T cells for immunotherapy of multiple advanced solid cancers
		Transposon-based non-viral vectors for improved CAR-T therapies

ESGCT 2025 Key Industry Sponsored Sessions Information (4/7)

Date	Sponsor	Title
08 Oct 2025	PlasmidFactory	Promises and challenges of in vivo CAR-T therapies
08 Oct 2025	Revvity	Next generation gene therapies for DMD
		OTOF – from basic research to gene therapy for hearing impairment
		In vivo CRISPR/Cas9 gene editing for ATTR amyloidosis and HAE - how close are we to getting these therapies to the market?
		Gene Therapy for Tay-Sachs and Sandhoff Diseas
08 Oct 2025	Sartorius	Walking the Tightrope | Balancing Process Optimization & Cost Implications to Improve CGT Accessibility
08 Oct 2025	Accuredit Therapeutics	Efficacy and safety of ART002, a single-dose gene editing therapy, in patients with severe heterozygous familial hypercholesterolemia

ESGCT 2025 Key Industry Sponsored Sessions Information (5/7)

Date	Sponsor	Title
09 Oct 2025	Vivet Therapeutics	VTX-PID (imlifidase) Mediated Optimal Depletion of Anti-AAV3B Neutralizing Antibodies in Human Subjects allows to Expand Patient Eligibility for future AAV-Based Gene Therapies : Results of a dose ranging and PK/PD modeling approach
09 Oct 2025	AskBio	RGX-202, an investigational gene therapy for the treatment of Duchenne muscular dystrophy: interim clinical data
		LUCE: Phase I/II Trial of Dual AAV Gene Therapy for Retinitis Pigmentosa in Usher Syndrome Type 1B
		RESET-PV: Initial clinical and translational data evaluating rese-cel (resecabtagene autoleucel), an autologous 4-1BBz CD19-CAR T cell therapy, without preconditioning, in pemphigus vulgaris
		GNT0004, Genethon's AAV-based gene therapy for Duchenne muscular dystrophy: long-term follow-up of ambulatory boys enrolled in the dose-escalation phase of GNT-016-MDYF
09 Oct 2025	Galapagos BV	The Galapagos Decentralized Cell Therapy Manufacturing Platform
09 Oct 2025	Cytiva	Large-scale transfection of T cells in a Xuri™ cell expansion system W25 using LNPs

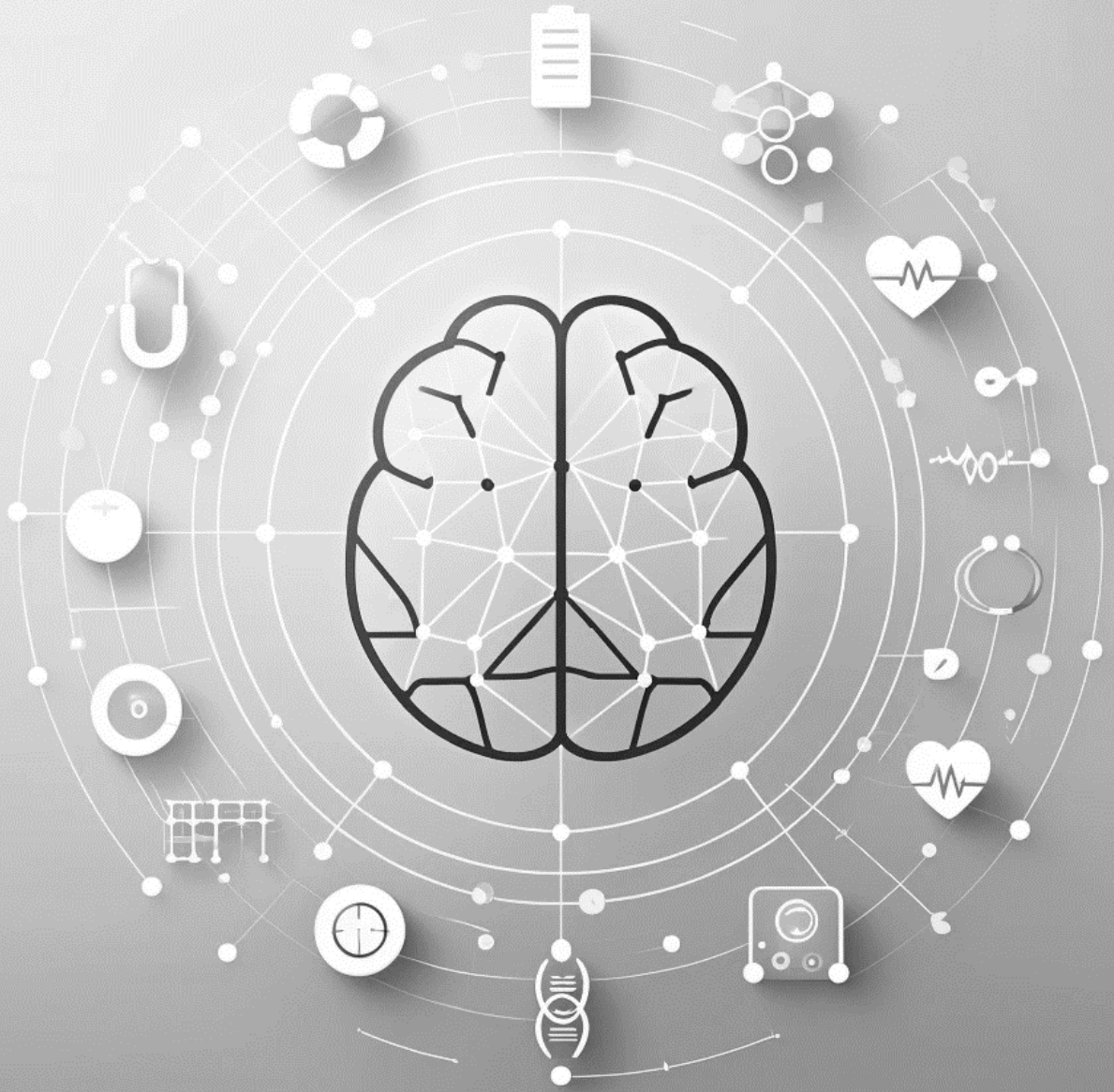
ESGCT 2025 Key Industry Sponsored Sessions Information (6/7)

Date	Sponsor	Title
09 Oct 2025	Roche	Transient blockade of the CD28/B7 costimulatory pathway allows AAV readministration by suppressing AAV vector and transgene immunity
09 Oct 2025	Repligen / Human Gene Therapy	Restoring Cell Health and Reversing Disease by Cellular Rejuvenation
		Gene Therapy for patients with Fanconi anemia: A stem cell disease
		Epitope engineering to enhance cancer immunotherapy, gene therapy and beyond
		In vivo lentiviral vector gene transfer into hematopoietic stem and progenitor cells
		Advancing AAV-based gene therapy for inherited retinal disorders due to mutations in large genes
09 Oct 2025	Merck	To 1000L and beyond: Innovating Upstream AAV manufacturing for the next wave of gene therapies

ESGCT 2025 Key Industry Sponsored Sessions Information (7/7)

Date	Sponsor	Title
09 Oct 2025	CSL Behring	Sustained disease correction is achieved without long-term vector-related liver toxicity four years after etranacogene dezaparvovec hemophilia gene therapy both in individuals with and without early transaminitis
09 Oct 2025	Astra Zeneca	In vivo Genome Editing with SpOT-On precision
10 Oct 2025	CSL Behring	AAV-based gene therapy for haemophilia B: the way to market authorization and future clinical development
10 Oct 2025	CRISPR-X, CRISPR Therapeutics	Single-dose in-vivo gene correction of AATD via LNP-delivered SyNTase editors
10 Oct 2025	EuroGCT	European Union policies and their impact on ATMP Research and Innovation
		The 2022 French Strategy on biotherapies and bioproduction of advanced therapies: results after 3 years launch and perspectives
		Support for ATMPs development in Spain: the central role of the Spanish Medicines Agency (AEMPS)

Noteworthy AI / ML presentations at ESGCT 2025



Themes from key AI / ML presentations at ESGCT 2025 (1/3)

- **ESGCT 2025 is expected to see AI and machine learning set to become integral to every stage of gene therapy, from vector design and delivery optimization to manufacturing control and biomarker analytics, accelerating precision and scalability across therapeutic platforms**
- Check out the key AI / ML themes at ESGCT 2025 below:
- **AI-Enabled Vector Engineering:**
  - AI systems are expected to redefine AAV and LNP vector design, integrating data-driven capsid prediction and rational optimization for tropism, immune evasion, and improved yield across translational programs
- **Precision Promoter and Payload Design:**
  - Tools such as PromoterGPT are projected to accelerate the generation of cell-type-specific promoters, improving gene expression control and minimizing off-target transcription in therapeutic constructs
- **AI-Integrated Manufacturing & Process Analytics:**
  - AI-based holographic imaging and LLM-driven knowledge-graph analytics are likely to enhance real-time process control, enabling predictive bioreactor performance and quality assurance in large-scale production.

Themes from key AI / ML presentations at ESGCT 2025 (2/3)

- **Computational Target Discovery & Validation:**
  - In-silico models are anticipated to streamline microglia-specific and tissue-selective target discovery, bridging preclinical validation with rapid AI-assisted prediction pipelines for vector-disease matching
- **AI-Optimized Delivery Platforms:**
  - Studies combining AI-guided LNP and polymer-based nanoparticle systems are expected to advance precision siRNA and mRNA delivery, particularly in macrophage modulation and CFTR gene restoration
- **Generative AI in Capsid Innovation:**
  - Generative algorithms, exemplified by GMU037, are predicted to identify dual-fitness capsids achieving superior ophthalmic transduction and scalable high-yield production for clinical translation
- **AI-Assisted Immune & Oncologic Therapeutics:**
  - Machine-learning-driven T-cell receptor design is set to overcome therapeutic resistance in myeloma, while AI-guided AAV vectors target hepatocellular carcinoma with improved selectivity and reduced immunogenicity

Themes from key AI / ML presentations at ESGCT 2025 (3/3)

- **Predictive Biomarker Quantification:**
  - AI-based quantification of COL4A5 in Alport syndrome is expected to refine therapeutic thresholds, linking histological biomarkers with gene-therapy dose–response correlations in upcoming clinical frameworks
- **Digitalization of Gene-Therapy Pipelines:**
  - The emergence of web-based AI applications for downstream analytics will strengthen closed-loop automation and accelerate process understanding across viral vector manufacturing chains
- **Synergy of AI and Genome Editing:**
  - AI-designed adenine deaminases integrated into the Pin-point™ base-editing platform are projected to expand precision editing, increasing correction efficiency while reducing error rates in early-phase pipelines

Noteworthy AI / ML presentations at ESGCT 2025

Notable Presentations Information At ESGCT 2025
AI / ML (1/3)

Date	Author	Title
08 Oct 2025	Hiroyuki Nakai	Defining therapeutic thresholds in AAV gene therapy for Alport syndrome via AI-based quantification of COL4A5 deposition in the glomerular basement membrane
08 Oct 2025	Camille Alliot	Revolutionizing rAAV Design: AI-Guided Rational Strategies Achieve Target-Specificity Binding
08 Oct 2025	SUZUKA HARA	Sequential Selection of Cell- or Tissue-Tropic AAV Variants Using an AI-driven NAb-Evading Library
08 Oct 2025	Adele De Hoffer	PromoterGPT: AI-Driven Design of Cell-Type-Specific Promoters
08 Oct 2025	Gabriele Pelosi	AI-driven chimeric T-cell receptors to tackle therapeutic resistance in myeloma.
08 Oct 2025	Guillaume Godefroy	Advancing bioreactor monitoring: holographic imaging device and AI-assisted image processing pipeline for cell viability measurement with an at-line probe

Notable Presentations Information At ESGCT 2025
AI / ML (2/3)

Date	Author	Title
08 Oct 2025	Xianqing Wang	AI-Driven Optimization of Hyperbranched Poly(amino ester)s for CFTR Gene Delivery in a Cystic Fibrosis Mouse Model
08 Oct 2025	Achim Aigner	Polymer-based nanoparticles for siRNA delivery into macrophages: RNAi-mediated STAT3/STAT6 knockdown allows M1 (re-)polarization
09 Oct 2025	Deborah Nazareth	Rational bioengineering of tumour-selective AAV vectors using one-shot AI for targeted gene therapy in hepatocellular carcinoma
09 Oct 2025	Killian Hanlon	Engineering optimised AAV capsids via AI-guided in silico design
09 Oct 2025	Sourav Choudhury	Generative AI Discovers GMU037, a Dual-Fitness Capsid with Simultaneous Superior NHP Ophthalmic Transduction and High Production Yield
09 Oct 2025	Alix Silvert	In-silico Discovery and Experimental Validation of Microglia-Specific Targets for AI-Based AAV Vector Design

Notable Presentations Information At ESGCT 2025
AI / ML (3/3)

Date	Author	Title
09 Oct 2025	Pablo Perez-Duran	Synergizing the Pin-point™ base editing platform with AI-designed adenine deaminases
09 Oct 2025	Yanis Habtoun	A Web App for Viral Vector Production Downstream Processing Analytics by Knowledge Graph and LLM Technology
09 Oct 2025	Maya Sugden	Engineering AI-guided LNPs for cell-specific mRNA delivery

Strategic Insights and Strategy Development is our focus